HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

HOUSEKEEPING

1. **Please pay 2022 dues today!**
   Thank you to the patient groups that have already paid.
   Help us reach our 100% participation goal!

Pay dues

2. **Have you given Haystack a list of how many patients you have in each state? Not patient names/addresses, but simply how many in TX, CA, etc. Your fellow patient groups have benefitted from Haystack keeping an aggregated list and are now helping us coordinate. Learn more about why this is such a critical tool for all our patient groups and share your data!**

Email Kara

3. **We will not have a Speaker (2nd Thurs each month), our standing monthly call (3rd Thurs each month) or our Work Group calls in Aug in light of our staff and patient groups' travel and much needed (!) summer vacation schedules.**

4. **Two articles we thought would be of interest:**

Article #1 **Article #2**

ADVOCACY MANAGER NEEDED!

For a complete list of current responsibilities, please visit the job posting below.

View posting

Please submit resumes to
Haystack1@HaystackProject.org

LEGISLATIVE UPDATE

DISCUSSION THIS MONTH CENTERED AROUND:

We discussed provisions on both the UFA package and the Drug Pricing bill relevant to our community, including:

- **Heart Act**
- **Accelerated Approval Pathway**
- **Orphan exemption from direct price negotiation**

We focused on understanding the policies and how we might add our voices to the education efforts needed for Senate HELP and Finance committees appreciate our concerns.

REGULATORY UPDATE

CMS has released both the Physician Office and Hospital Outpatient proposed rules. We reviewed and discussed examples of impact of several agency proposals, including:

- **Bonus for underserved ACO patients**
- **Remote therapeutic monitoring codes**
- **High value under-utilized services**

HHS also released a Request for Information on Improving Primary Care Services. We discussed the need to educate primary care doctors, especially pediatricians, to improve their approach to rare diseases.

Comment letters on both forthcoming.

SPEAKER SERIES

This month, Haystack hosted
Dr. Joni Rutter and Dr. Eric Sid

Haystack members had requested an opportunity to better understand the research related reasons to have an ICD-10 code. We've never had a bigger audience attend (!) and the discussion was thoughtful and in-depth and the conclusion remarkable. By the time we addressed the various aspects of ICD-10 codes and research, Dr. Rutter agreed with the following summation:

Joni: "So we can't give up on ICD10 codes because they serve the purpose of public health and payers, but from the purpose of research, because we keep hearing that it's tied to finding patients for research, *it's just not that helpful yet.* And I don't want to make that statement if it isn't loud and clear what you're saying. It's almost become a "rallying cry" of ultra-orphan diseases and it pushes a button in us because we're so tired of not knowing that anyone else knows about the disease. But if you spend enough time understanding how doctors and insurance companies use ICD10 codes, you begin to pause, and you begin to say 'I better understand before I just do it because it's the national cry. I better just figure out with my doctors, with my patients, with the treatments they take for symptoms management or side effects that they need today - the treatment may be 10 years away but patients are taking stuff right now and there are [existing] codes that identify them right now....' So i think what we've been saying at Haystack Project is -- *let's make sure that your particular circumstances warrant a code*. Don't just go do it because it's a 'rallying cry.'

Saira: I want to ask you very directly, I'm hearing you say it's not the end all and be all of research, and we need you to think about these other circumstances before you do it because what we really need [for research] is not this narrow static code, we need to be facile and ask broader questions and get a handle on symptoms.

Dr. Rutter's response was succinct:

"Well said."

We then explored what the 'rallying cry' should be instead of ICD-10 codes if we are truly to help with research.

Joni: "I don't know that there's any one thing that we can point to. There are a variety of activities in this space are ongoing. I mentioned the human phenotype in oncology and other types of registries and CPATH. It may be worth doing a landscape analysis of these registries to see how they're thinking about this and see how this group might pull it together and coordinate across the registries. There's a variety of activities you think about...worth looking into other registries and what they're doing."

The full discussion will be posted to Haystack's website.

WORKGROUPS

HP50/State Activities

(led by Dean Suhr, MLD Foundation)

The work group has landed on an approach for supporting HP patient orgs with capacity building in the states in ways that align with HP's mission. The work group will take a pilot approach with 1-2 patient groups in 1-3 states. We discussed the "profile" of what kind of patient groups could make for a successful pilot and asked groups to reach out to the work group with interest.

If any patient groups want to participate, please email haystack@haystackproject.org and include "HP50 pilot" in the subject line if your group aligns with the profile.

Meets second Tuesdays at 3pm ET

Email Dean

HEART & Accelerated Approval

(led by Kara Berasi, CDG Cares and Dottie Caplan, Applied Therapeutics)

The work group is actively searching for patients, educating them, and supporting them through the process of communicating with Senate HELP Committee senators on the communities:

- need for the Heart Act provisions
- ·support for the oversight/consistency role of the Accelerated Approval (AA) Pathway Council, and
- ·interest in the mismatch of including the Office of Orphan Products rather than the rare review division on the AA Pathway Council.

If any patient groups want to meet with Senator Kaine (D-VA) about the Accelerated Approval issues, please contact Kara Berasi (kkberasi@gmail.com).

We also discussed the political dynamics, timing, etc. of the overall FDA UFA package.

Meets second Mondays at 3pm ET

Email Kara

HR 6160 / Medical Necessity

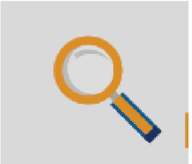
(led by Marc Yale, IPPF)

The work group is focused this month on arranging patient/physician meetings with Members of Congress during recess. If you are a patient, patient group, caregiver, etc. in Michigan, Pennsylvania or California, please contact Marc at marc@pemphigus.org.

Marc also previously asked to chat with anyone already participating or interested in EveryLife's Rare Across America meetings in these or other states.He is interested in making sure you have information about HR 6160, a bill to help address the constant denials patients get from insurance for the care their doctors prescribe.

Meets first Wednesdays at 4:30pm ET

Email Marc



First Look Workgroup

(led by Michelle Davis, IFOPA and Julie Boyd, Ultragenyx)

This work group just met with Tina Iirv, spelling, title? to learn what might already exist by way of clinically observable initial signs of rare diseases. HP is pursuing several leads suggested by both Tina and this work group members.

Please contact haystack@haystackproject if you would like to join this work group and learn more about this effort or if you have/represent a rare condition that has some initially observable signs that warrant further inquiry for diagnosis.

Meets third Wednesdays at noon ET.

Email Michelle

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Haystack Project is grateful to all of our Alliance Partners for lending their insights and perspectives, as well as for combining their efforts with ours to better serve the rare and ultra-rare communities.

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